

IN THE UNITED STATES PATENT AND TRADEMARK OFFICE

In re Applicant:

FUTERMAN Anthony et al

Serial No.: 10/552,287

Filed: April 18, 2004

For: GAUCHER DISEASE DRUGS AND
METHODS OF IDENTIFYING SAME

Examiner: Not Yet Assigned

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Group Art Unit: 1656

Attorney
Docket: 30227

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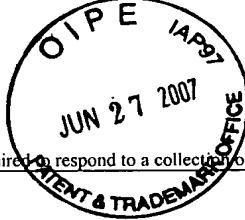
Enclosed is a PTO Form 1449 which lists citations which may be material to the patentability and examination of the above identified application. Also enclosed are copies of the references cited. These are submitted in compliance with the duty of disclosure defined in 37 CFR 1.56. The Examiner is requested to make these citations of official record in this application.

This Information Disclosure Statement under 37 CFR 1.56 is not to be construed as a representation that a search has been made, that additional matter which is material to the examination of this application does not exist, or that any or more of these citations constitutes prior art.

Respectfully submitted,

Martin D. Moynihan
Registration No. 40,338

Dated: June 18, 2007



PTO/SB/08b (08-03)

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**INFORMATION DISCLOSURE
STATEMENT BY APPLICANT**

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Application Number	10/552,287
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Sheet	1	Of	4
OTHER PRIOR ART – NON PATENT LITERATURE DOCUMENTS			
Examiner Initials	Cite No. ¹	Include name of the author (in CAPITAL LETTERS), title of the article (when appropriate), title of the item (book, magazine, journal, serial symposium, catalog, etc.) date, page(s), volume-issue number(s), publisher, city and/or country where published.	T ²
	1	Grabowski et al. "Human Acid β -Glucosidase - Use of Conduritol B Epoxide Derivatives to Investigate the Catalytically Active Normal and Gaucher Disease Enzymes", The Journal of Biological Chemistry, 261(18): 8263-8269, 1986.	
	2	Berg-Fussman et al. "Human Acid β -Glucosidase - N. Glycosylation Site Occupancy and the Effect of Glycosylation on Enzymatic Activity", The Journal of Biological Chemistry, 268(20):14861-14866, 1993.	
	3	Roeber et al. "Crystallization and Preliminary X-Ray Analysis of Recombinant Human Acid Beta-Glucocerbrosidase, A Treatment for Gaucher's Disease", Biological Crystallography, D59: 343-344, 2003.	
	4	Dvir et al. "X-Ray Structure of Human Acid- β - Glucosidase, the Defective Enzyme in Gaucher Disease", The EMBO Journal, P.1-27, 2003.	
	5	Sawkar et al. "Chemical Chaperones Increase the Cellular Activity of N370S β -Glucosidase; A Therapeutic Strategy for Gaucher Disease", PNAS, 99(24): 15428-15433, 2002.	
	6	Erickson et al. "Biosynthesis of the Ltsomal Enzyme Glucocerebrosidase", The Journal of Biological Chemistry, 260(26): 14319-14324, 1985.	
	7	Ahn et al. "Crystal Structure of Saposin B Reveals A Dimeric Shell for Lipid Binding", Proc. Natl. Acad. Sci. USA, 100(1): 38-43, 2003.	
	8	Amaral et al. "Gaucher Disease: Expression and Characterization of Mild and Severe Acid β -Glucosidase Mutations in Portuguese Type 1 Patients", European Journal of Human Genetics, 8: 95-102, 2000.	
	9	Amaral et al. "Type 1 Gaucher Disease: Identification of N396T and Prevalence of Glucocerebrosidase Mutations in the Portuguese", Human Mutation, 8: 280-281, 1996.	
	10	Beutler "Economic Malpractice in the Treatment of Gaucher's Disease", The American Journal of Medicine, 97: 1-2, 1994.	
	11	Beutler et al. "Gaucher Disease", The Metabolic and Molecular Bases of Inherited Disease, Chap.146: 3635-3668, 2001.	
	12	Beutler et al. "Two New Gaucher Disease Mutations", Human Genetics, 93: 209-210, 1994.	
	13	Brünger et al. "Crystallography & NMR System: A New Software Suite for Macromolecular Structure Determination", Acta Crystallographica Section D, 54: 905-921, 1998.	
	14	Buccoliero et al. "The Role of Sphingolipids in Neural Development: Lessons From Models of Sphingolipid Storage Diseases", Neurochemical Research, 27(7/8): 565-574, 2002.	

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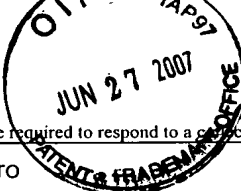
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Group Art Unit	1656
Examiner Name	Not Yet Assigned

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Sheet	2	Of	4
OTHER PRIOR ART – NON PATENT LITERATURE DOCUMENTS			
	15	Charrow et al. "The Gaucher Registry. Demographics and Disease Characteristics of 1698 Patients With Gaucher Disease", Archive of Internal Medicine, 160: 2835-2843, 2000.	
	16	Chi et al. "Crystal Structure of the β -Glycosidase From the Hyperthermophile Thermosphaera Aggregans: Insights Into Its Activity and Thermostability", FEBS Letters, 445: 375-383, 1999.	
	17	Cox et al. "Novel Oral Treatment of Gaucher's Disease With N-Butyldeoxynojirimycin (OGT 918) to Decrease Substrate Biosynthesis", The Lancet, 355: 1481-1485, 2000.	
	18	Davies et al. "Structures and Mechanisms of Glycosyl Hydrolases", Structure, 3: 853-859, 1995.	
	19	Dinur et al. "Human Acid β -Glucosidase: Isolation and Amino Acid Sequence of A Peptide Containing the Catalytic Site", Proc. Natl. Acad. Sci. USA, 83: 1660-1664, 1986.	
	20	Fabrega et al. "Site Actif de la Glucocérébrosidase Humaine: Prédiction Structurales et Validations Expérimentales", Journal de la Société de Biologie, 196(2): 151-160, 2002. Article in French.	
	21	Fabrega et al. "Human Glucocerebrosidase: Heterologous Expression of Active Site Mutants in Murine Null Cells", Glycobiology, 10(11): 1217-1224, 2000.	
	22	Fan "A Contradictory Treatment for Lysosomal Storage Disorders: Inhibitors Enhance Mutant Enzyme Activity", Trends in Pharmacological Sciences, 24(7): 355-360, 2003.	
	23	De La Fortelle et al. "Maximum-Likelihood Heavy-Atom Parameter Refinement for Multiple Isomorphous Replacement and Multiwavelength Anomalous Diffraction Methods", Methods in Enzymology, 276: 472-494, 1997.	
	24	Futerman et al. The Cell Biology of Lysosomal Storage Disorders", Nature Reviews in Molecular & Cellular Biology, 5: 554-565, 2004.	
	25	Futerman et al. "New Directions in the Treatment of Gaucher Disease", Trends in Pharmacological Sciences, 25(3): 147-151, 2004.	
	26	Grabowski et al. "Enzyme Therapy for Lysosomal Storage Disease: Principles, Practice, and Prospects", Annual Reviews in Genomics & Human Genetics, 4: 403-436, 2003.	
	27	Grabowski et al. "Enzyme Therapy in Type I Gaucher Disease: Comparative Efficacy of Mannose-Terminated Glucocerebrosidase From Natural and Recombinant Sources", Annals of Internal Medicine, 122(1): 33-39, 1995.	
	28	Grabowski et al. "Acid β -Glucosidase: Enzymology and Molecular Biology of Gaucher Disease", Critical Reviews in Biochemistry and Molecular Biology, 25(6): 385-414, 1990.	
	29	Grace et al. "Analysis of Human Acid β -Glucosidase by Site-Directed Mutagenesis and Heterologous Expression", The Journal of Biological Chemistry, 269(3): 2283-2291, 1994.	

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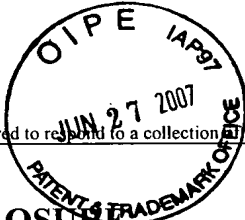
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34	Jones "A Graphics Model Building and Refinement System for Macromolecules", Journal of Applied Crystallography, 11: 268-272, 1978.		
35	Jones et al. "Improved Methods for Building Protein Models in Electron Density Maps and the Location of Errors in These Models", Acta Crystallographica Section A, 47: 110-119, 1991.		
36	Korkotian et al. "Elevation of Intracellular Glucosylceramide Levels Results in An Increase in Endoplasmic Reticulum Density and in Functional Calcium Stores in Cultured Neurons", The Journal of Biological Chemistry, 274(31): 21673-21678, 1999.		
37	Lachmann "Miglustat Oxford GlycoSciences/Actelion", Current Opinion in Investigational Drugs, 4(4): 472-479, 2003.		
38	Legler "Glucosidases", Methods in Enzymology, 46(Chap.40): 368-381, 1977.		
39	Legler "Glycoside Hydrolases: Mechanistic Information From Studies With Reversible and Irreversible Inhibitors", Advances in Carbohydrate Chemistry and Biochemistry, 48: 319-384, 1990.		
40	Lloyd-Evans et al. "Glucosylceramide and Glucosylsphingosine Modulate Calcium Mobilization From Brain Microsomes Via Different Mechanisms", The Journal of Biological Chemistry, 278(26): 23594-23599, 2003.		
41	Meivar-Levy et al. "Analysis of Glucocerebrosidase Activity Using N-(1-[¹⁴ C]Hexanoyl)-D-Erythro-Glucosylsphingosine Demonstrates A Correlation Between Levels of Residual Enzyme Activity and the Type of Gaucher Disease", Biochemical Journal, 303: 377-382, 1994.		
42	Miao et al. "Identification of Glu340 as the Active-Site Nucleophile in Human Glucocerebrosidase by Use of Electrospray Tandem Mass Spectrometry", The Journal of Biological Chemistry, 269(15): 10975-10978, 1994.		
43	Mistry et al. "Therapeutic Delivery of Proteins to Macrophages: Implications for Treatment of Gaucher's Disease", The Lancet, 348: 1555-1559, 1996.		
44	Morel et al. "Effect of Mutations Within the Peripheral Anionic Site on the Stability of Acetylcholinesterase", Molecular Pharmacology, 55: 982-992, 1999.		

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